A suite of engineered human pluripotent stem cell lines to facilitate the generation of hematopoietic stem cells

Grant Award Details

A suite of engineered human pluripotent stem cell lines to facilitate the generation of hematopoietic stem cells

Grant Type: Tools and Technologies III

Grant Number: RT3-07763

Project Objective: To create a suite of human ESC reporter lines that can be used to monitor key milestones in HSC biology, and will serve as a research tools for developing authentic HSCs for therapeutic applications.

Investigator:

Name: Hanna Mikkola

Institution: University of California, Los Angeles

Type: PI

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Name: Andrew Elefanty

Institution: Blood Cell Development and Disease Murdoch Childrens Research Institute The Royal Children's Hospital

Type: Partner-PI

Disease Focus: Blood Disorders

Collaborative Funder: Australia

Human Stem Cell Use: Embryonic Stem Cell

Cell Line Generation: Embryonic Stem Cell

Award Value: $1,382,400

Status: Active

Progress Reports

Reporting Period: Year 1

View Report

Grant Application Details

Application Title: A suite of engineered human pluripotent stem cell lines to facilitate the generation of hematopoietic stem cells

Public Abstract: Our goal is to develop tools that address major bottlenecks that have prevented the generation of blood forming stem cells in culture for therapeutic use. To help overcome these bottlenecks, we will generate a suite of human embryonic stem cell reporter lines that can be used to monitor key milestones in blood stem cell development. These lines will serve as tools to identify factor combinations to improve the in vitro differentiation of hESCs to functional blood stem cells. Once individual lines have been validated, lines that contain multiple fluorescent reporters will be generated, and a multi factor screen will be performed to optimize conditions that induce these blood stem cell regulators. To track the location and quantity of transplanted cells in recipient small animal model, we will generate hESC lines with in vivo reporter system that combines bioluminescent or PET imaging, and serum-based assay. Our in vivo tracking tools will be broadly relevant and not restricted to studying the in vivo biology of blood forming cells. These tools will help translate the promise of stem cells to cell based therapies to treat human disease.

Statement of Benefit to California: This project will help improve California economy as many of the vendors used for reagents and supplies are located in California. This project will also help create and maintain jobs for skilled personnel and helps train post-doctoral fellows who will become the next generation of stem cell scientists. The long-term goal of this project is to improve in vitro differentiation protocols to create transplantable blood forming stem cells for therapeutic use. If we, or others who will use our reporter lines generated in this study, achieve this goal, there will be new, theoretically unlimited sources of HLA-matched or patient specific blood stem cells that can be used for treating many serious blood diseases, including leukemias and inherited immunodeficiencies or anemias. Availability of patient specific blood stem cells for transplantation would be a major benefit in California, as there is currently limited availability of suitable bone marrow donors for individuals from mixed ethnic backgrounds.